Proposed Decision Memo for Stem Cell Transplantation (CAG-00287R)

Decision Summary

The Centers for Medicare and Medicaid Services (CMS) proposes the following:

Stem cell transplant and high dose chemotherapy are both integral to the course of treatment and covered as a single entity.

Back to Top

Proposed Decision Memo

TO: Administrative File: CAG-00287R

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SUBJECT: Stem Cell Transplantation

DATE: August 30, 2005

I. Proposed Decision

The Centers for Medicare and Medicaid Services (CMS) proposes the following:

Stem cell transplant and high dose chemotherapy are both integral to the course of treatment and covered as a single entity.

II. Background

On April 20, 2005, CMS began a reconsideration of the current NCD for stem cell transplantation to review the definition of stem cell transplantation.

Contemporaneously with our last decision on stem cell transplantation in March 2005, CMS became aware of instances in which confusion arose as to the appropriate reimbursement for stem cell transplantation. In some instances, the stem cell transplantation was nationally noncovered but contractors were required to provide coverage for the initial high dose chemotherapy. CMS has elected to internally generate this NCD to address this discrepancy.

In Board of Trustees of University of Arkansas v. Secretary of HHS, 354 F.Supp.2d 924 (E.D. Ark.2005), the court determined that the applicable NCD language and Manual provisions excluded from coverage autologous stem cell transplant, but supported the coverage of high dose chemotherapy. This finding is troubling because high dose chemotherapy causes significant toxicity that would not be undertaken without a companion remedy offered by the replacement of stem cells. The intent of the stem cell transplant NCD was to cover or not cover both high dose chemotherapy and stem cell transplant as a single treatment strategy.

Stem Cell transplantation (Autologous or Allogeneic) is a treatment strategy that includes a number of stages over a period of weeks. The first stage is called mobilization where the patient is given a granulocyte colony-stimulating factor (G-CSF) or a granulocyte-macrophage colony-stimulating factor (GM-CSF) to stimulate the release of the stem cells from storage sites within the body. Next, the stem cells are harvested via leukapheresis or bone marrow biopsy. The next stage is called conditioning where the patient is given a high dose of a chemotherapy agent or radiotherapy. In the final stage the harvested stem cells are administered along with supportive medical care.

III. History of Medicare Coverage

Medicare is a defined benefit program. An item or service must fall within a benefit category as a prerequisite to Medicare coverage. §1812 (Scope of Part A); §1832; (Scope of Part B); §1861 (s) (Definition of Medical and Other Health Services).

CMS has determined that autologous and allogeneic stem cell transplantation falls within the benefit category of inpatient hospital services under Part A and physicians' services under Part B. See §1812 (a)(1) (inpatient hospital services); §1832 (outpatient hospital services incident to a physician's service); §1861(s)(2) (incident to physician's services); §1861(b) (inpatient hospital services).

Section 110.8.1 of the National Coverage Determination (NCD) Manual

(http://www.cms.hhs.gov/manuals/103_cov_determ/ncd103c1_Part2.pdf) lists those instances in which autologous or allogeneic stem cell transplantation are nationally covered, nationally noncovered or left to contractor discretion. CMS last reviewed specific indications for autologous stem cell transplantation in March 2005.

IV. Timeline of Recent Activities

On April 20, 2005 CMS opened this reconsideration of the current NCD for stem cell transplantation to review the definition of stem cell transplantation.

On May 20, 2005, initial public comments were posted to the tracking sheet available at http://www.cms.hhs.gov/mcd/viewpubliccomments.asp?nca_id=159.

V. FDA Status

Stem cell transplantation is regulated under 21 C.F.R. §1271.3, which distinguishes between autologous and allogeneic stem cell transplantation. Section 1271.3(a) defines the term autologous use as “the implantation, transportation, infusion, or transfer of human cells or tissue back into the individual from whom the cells or tissue were recovered.” Section 1271.3(c) defines the term homologous use as “the replacement or supplementation of a recipient`s cells or tissues with an HCT/P that performs the same basic function or functions in the recipient as in the donor.”

Section 1271.3(d) defines human cells, tissues, or cellular or tissue-based products (HCT/P’s) as “articles containing or consisting of human cells or tissues that are intended for implantation, transplantation, infusion or transfer into a human recipient. Examples of HCT/P’s include, but are not limited to, bone, ligament, skin, dura mater, heart valve, cornea, hematopoietic stem cells derived from peripheral and cord blood, manipulated autologous chondrocytes, epithelial cells on a synthetic matrix, and semen or other reproductive tissue.”

VI. Evidence

1. Public Comments

A. Initial 30-Day Comment Period

CMS received four initial public comments. Three comments suggest a more comprehensive definition of stem cell transplantation, including providing coverage for the outpatient portions of treatment and reflecting the entire set of services necessary for the procedure. In response, these comments are outside the scope of this reconsideration. One commenter suggests financial incentives for doctors to perform as much of the treatment in the outpatient setting as possible. The commenter notes that currently, CMS offers incentives for inpatient transplant and penalizes programs that can perform transplants in the outpatient setting. Again, this comment is outside the scope of this NCD. One commenter indicates that it would be desirable to separate hematopoietic stem cell acquisition from the transplant itself. For autologous stem cell acquisition this should encompass either bone marrow harvest or peripheral blood stem cell collections. Allogeneic stem cell acquisition should include bone marrow, peripheral blood stem cell and umbilical cord blood as all are standard sources of hematopoietic stem cells. In response, these are all payment issues which were not part of the NCD process and should be addressed elsewhere in the Agency.

All four commenters suggest expanding coverage of stem cell transplants by adding indications including: myelodysplastic syndrome, non-Hodgkin’s lymphoma, multiple myeloma, tandem transplant in myeloma, myelofibrosis, relapsed or refractory non-Hodgkins, relapsed or refractory Hodgkins Lymphoms, chemotherapy/medication induced irreversible aplasia, or high risk multiple myeloma. One commenter indicates that efficacy of tandem transplant in myeloma when a complete response is not achieved after the first transplant is proven by the French Myeloma group in NEJM. CMS notes that these comments relate to the expansion of coverage for stem cell transplants, which is not addressed in this NCD, and welcomes commenters to request that CMS open a separate NCD to address expansion of coverage.

VII. CMS Analysis

National coverage determinations (NCDs) are determinations by the Secretary with respect to whether or not a particular item or service is covered nationally under title XVIII of the Social Security Act § 1869(f)(1)(B). In order to be covered by Medicare, an item or service must fall within one or more benefit categories contained, and must not be otherwise excluded from coverage. Moreover, with limited exceptions, the expenses incurred for items or services must be "reasonable and necessary for the diagnosis or treatment of illness or injury or to improve the functioning of a malformed body member." § 1862(a)(1)(A).

The early treatment of certain cancers and hematologic disorders focused on oral chemotherapy regimens, first with standard doses of drugs such as melphalan, prednisone, and colchicines, and later with multiple drug regimens. However, certain conditions respond poorly to standard chemotherapy, and many patients do not benefit from this treatment. The poor response rates experienced with only chemotherapy prompted the development of high dose chemotherapy with (autologous or allogeneic) stem cell transplant. This treatment strategy includes a number of stages over a period of weeks. The first stage is called mobilization where the patient is given a granulocyte colony-stimulating factor (G-CSF) or a granulocyte-macrophage colony-stimulating factor (GM-CSF) to stimulate the release of the stem cells from storage sites within the body. Next, the stem cells are harvested via leukapheresis or bone marrow biopsy. The next stage is called conditioning where the patient is given a high dose of a chemotherapy agent or radiotherapy. In the final stage the harvested stem cells are administered along with supportive medical care. There are no circumstances in which the myelotoxic doses of chemotherapy would be given without the subsequent stem cell administration.

Currently section 110.8.1 of the National Coverage Determination (NCD) manual (CIM §35-30.1) states that stem cell transplantation is a "process in which stem cells are harvested from either a patient's or donor's bone marrow or peripheral blood for intravenous infusion. The transplant can be used to effect hematopoietic reconstitution following severely myelotoxic doses of chemotherapy (HDCT) and/or radiotherapy used to treat various malignancies. Allogeneic stem cell transplant may also be used to restore function."

VIII. Proposed Decision

The Centers for Medicare and Medicaid Services (CMS) proposes the following:

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Back to Top